2015 Strategic Priorities

Pediatric Leukemia and Lymphoma Steering Committee (PLLSC)

The overall goal of the NCTN is to perform definitive phase 3 trials. Pediatric phase 3 trials are inherently limited in number due to the thankfully small numbers of patients with specific cancer types. Hence it is essential that questions of therapy for pediatric phase 3 trials for childhood leukemias and lymphomas be thoughtfully prioritized based on compelling basic and translational science and based on clinical experience documenting the activity and the feasibility of the planned therapeutic intervention.

Acute Lymphoblastic Leukemia (ALL)

- 1) Evaluate novel immunotherapy approaches for selected patient populations.
- 2) Evaluate the addition of targeted therapies to standard chemotherapy for patient populations whose leukemia cells have activating mutations in targetable kinases, or actionable aberrancies in other pathways.
- 3) Evaluate treatment approaches that minimize long-term morbidity such as osteonecrosis, neurocognitive deficits, and second cancers; or that would be potentially more effective and carry less adverse side effects than allogeneic hematopoietic stem cell transplantation.
- **4)** Optimize pharmacologically rational approaches to further improve outcomes for average and high-risk patient populations.
- **5)** Collaborate with other NCTN groups to identify acute lymphoblastic leukemia subgroups of interest to evaluate new treatment approaches.

Myeloid Leukemia

- 1) Evaluate the addition of targeted therapies to standard chemotherapy for genomically-defined patient populations whose leukemia cells have potentially targetable mutations including, but not limited to, mutations that occur at substantial rates in both the pediatric and adult age range.
- **2)** Evaluate treatment approaches that reduce the acute and long-term morbidity of therapy for children with AML while maintaining or improving overall outcome.
- **3)** Evaluate novel immunotherapy and other approaches for high-risk patient populations that may be more effective and carry less adverse side effects than allogeneic hematopoietic stem cell transplantation.
- **4)** Evaluate special myeloid leukemia subgroups (e.g., APL, Down syndrome AML, and JMML) for which there are research opportunities and clinical needs.
- **5)** Collaborate with other NCTN groups to identify myeloid leukemia subgroups of interest to evaluate new treatment approaches.